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SEQUENCE LISTING

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Humphries, Peter
Kenna, Paul

<120> Genetic Suppression and Replacement

<130> MUR-003

<140> 09/155,708
<141> 1999-04-05

<150> PCT/GB97/00929
<151> 1997-04-02

<150> GB9606961.2
<151> 1996-04-02

<160> 34

<170> PatentIn version 3.1

<210> 1
<211> 617
<212> DNA
<213> Artificial Sequence

<220>
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<220>
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<222> (1)..(617)
<223> n is any nucleotide

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<400> 1
tcccttntgn tagattgcan nncccaataa aanaaggncg cgcttaaagg cttatcgaaa 60
ttaatacgac tcactatang gagaccaag cttagagtca tccagctgga gccctgagtg 120
gctgagctca ggccttcgca gcattcttgg gtgggagcag ccacgggtca gccacaaggg 180
ccacagccat gaatggcaca gaaggcccta acttctacgt gcccttctcc aatgcgacgg 240
gtgtggtacg cagccccttc gagtaccac agtactacct ggctgagcca tggcagttct 300
ccatgctggc cgcctacatg tttctgctga tcgtgctggg cttccccatc aacttctca 360
cgctctacgt caccgtccag cacaagaagc tgcgcacgcc totcaactac atcctggctc 420
aacctagccg tggtgaact cttcatggtc ctangtggct tcaccagcac ctctacanct 480
ctctgcatgg atactcgtct tcgggccac aggatgcaat tgganggctc tttgcacctg 540
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agaacaactc cgctccc

617

<210> 2
<211> 639
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<213> Artificial Sequence

<220>
<223> The human rhodopsin hybrid cDNA with a C-->G change at nucleotide
271

<220>
<221> misc_feature
<222> (1)..(639)
<223> n is any nucleotide

<400> 2
ggnnnnttgg gtcgcgcatt naagaactca nggncccgca gcattcttgg gtgggagcag 60
ctacgggtca gccacaaggg ccacagccat gaatggcaca gaangcccta acttctacgt 120
gcccttctcc aatgcgacgg gtgtggtacg cagccccttc gagtaccac agtactacct 180
ggctgagcca tggcagttct ccatgctggc cgcctacatg tttctgctga tcgtgctggg 240
cttccccatc aacttctca cgctctacgt gaccgtccag cacaagaagc tgcgcacgcc 300
tctcaactac atcctgctca acctanccgt ggntgaactc ttcattggtcc taggtggctt 360
caccancaac ctctanacct ctctgcatgg anacttcttc ttccggccca caggatgcaa 420
tttgaaggn ttctttaac acccgggggg ggaaaattgc ctgtggtcct tgggtggccg 480
gncancnaac ggtacttgtg gtnnttaanc cataaacaat tccgcttcgg gaaaaacatg 540
ccancntggg gtttcttca ctnggttang ggcnggctgc cccacccca atcccnggtn 600
gtcaantaat cccaagggn nantgncntt ttaaacaaa 639

<210> 3
<211> 686
<212> DNA
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<220>
<223> A human rhodopsin adRP mutation, a C-->T change at nucleotide 217

<220>
<221> misc_feature
<222> (1)..(686)
<223> n is any nucleotide

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<400> 3
nnnttagggn cggatgtcna tataagcaga nctctctggg ctaactaana agaaccact      60
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gcctgagctc agccacaagg gccacagcca tgaatggcac agaaagccct aacttctacg      180
tgcccttctc caatgcgacg ggtgtgttac gcagcctctt cgagtacca cagtactacc      240
tggttgagcc atggcagttc tccatgctgg ccgcctacat gtttctgctg atcgtgctgg      300
gcttccccat caacttctc acgtctctacg tcaccgtcca gcacaagaag ctgcgcacgc      360
ctctcaacta catcctgctc aacctanccg tggctgaact cttcatggtc ctangtggct      420
tcaccancac cctctacacc tctctgcatg gatacttcgt cttccgggcc acaggatgca      480
at ttggaagg cttctttgca ncctgggncg ggaaattgcc tgtngtcttg gtggtcctgg      540
ccatcaacng tacttggtgt ntnttaccca tnaacaattc cgctccggga aaacatgcac      600
atgggn ttgc ctactnggt ctggggcngg cccccaccc ccccccggt ggtcanttat      660
cccangcgcn aatgcctttn annaaa                                           686

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<210> 4
<211> 787
<212> DNA
<213> Artificial Sequence

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<220>
<223> A hammerhead ribozyme (termed Rz10) cloned in pCDNA3

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<220>
<221> misc_feature
<222> (1)..(787)
<223> n is any nucleotide.

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<400> 4
cngcncgttg aaatataagc agaccctctg gntaactana ataaccactg cttactggct      60
tatcgaaatt aatacgactc actatangga gaccaagctt ggtcggctctg atgagtcctg      120
gaggacgaaa cgtagagtct anagggccct attctatagt gtcacctaaa tgctaganct      180
cgctgatcag cctcgactgt gccttctagt tgccagccat ctgttggttg cccctcccc      240
gtgccttctc tgancctgga aggtgccact cccactgtcc tttcctaata aaatgagnaa      300
ttgcntctca ttgtctgagt agtgtcatcc aatctggggg tgggtggggc agnacacnag      360
gggaagatgg gaaaacatac aggcattgctg gggangccgt ggntctatgn ctngaggcg      420
aaaaaacact ggggnctagg ggtacccac cccctgtacg gccataacnc gnggtttgtg      480

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gtacccta acgtanntgc accctaccg ncttcttct cctcttncca ttccgggtc 540
cctcaccnaa cgggccttng tcatactng gnccaccaa tanagtagtc ttgccccca 600
aagtcctna tgacctntaa gaccttcann anccccctt nttnaaana ncnnnnnnn 660
nnnnnnnnnc cngnaaaan aacaactaat ttgggaacc ccccccnaa aacccttcc 720
ntntcccccc nattaatnt tnnntnccc cccccccc cccnntttt tnnccccn 780
nnannng 787

<210> 5
<211> 665
<212> DNA
<213> Artificial Sequence

<220>
<223> A hammerhead ribozyme (termed Rz20) cloned in pCDNA3

<220>
<221> misc_feature
<222> (1)..(665)
<223> n is any nucleotide.

<400> 5
nnccccgcc ntttnaaana anccnagcct ctggcnaact ananaaccac tgcttactgg 60
cttatcnaaa ttaatacgac tcactatagg gagaccaag ctttactga actgatgagt 120
ccgtgaggac gaaaggctgc tctananggc cctattctat antgtcacct aaatgctaga 180
gctcgctgat cagcctcgac tgtgccttct aattgccage catctgttgt ttgccctcc 240
cccgctcctt ccttgaccct ggaagggtgcc actccactg tcctttccta ataaaatgaa 300
gatnttncat cncattgtct gagtaagtgt cattctattc tggggggtgg ggtggggcac 360
gacancaang gggaagattg ggaaaaata ncaggcntgc tggggatncc gtgggctcta 420
tngcttctga agcggaaaaa acaactgggg ctctangggg tatccccccc ccctgtaac 480
gngcattaaa cncgggggtg ttgtggttac cccaacttaa cgctancttg caacgccna 540
acgccccncc ttctcttct ccttccttc ncccacttc cgggttccn tcaaccnnaa 600
tcggggcccc ttaggtccaa ttatgcttcg gcccncnccn aaactaatag gtnggttctt 660
tngcc 665

<210> 6
<211> 624
<212> DNA
<213> Artificial Sequence

<220>
 <223> Mouse rhodopsin cDNA cloned into pCDNA3

<220>
 <221> misc_feature
 <222> (1)..(624)
 <223> n is any nucleotide.

<400> 6
 nnnncttncct tanngcttgg taccganctc ggatccacta gtnaacggcc gccagtgtgc 60
 tggaaattcc cagaggnact ctggggcaga caagatgaga caccctttcc tttctttacc 120
 taagggcctc caccgatgt caccttggcc cctctgcaag ccaattaggc cccggtggca 180
 gcagtgggat tagcgtagt atgatatctc gcggatgctg aatcagcctc tggcttaggg 240
 agagaaggtc actttataag ggtctggggg gggtcagtgc ctggagttgc gctgtgggag 300
 ccgtcagtgg ctgagctcgc caagcagcct tggctctgt ctacgaaan cccgtggggc 360
 agcctcnana accgcagcca tgaacggcac agaaggcccc aatttttatg tgcccttctc 420
 caacgtcaca ngcgtggtgc ggaaccctt cnancanccg cagtactacc tggcggaacc 480
 atggcagttc tccatgctgg canctacat gtctgtctca tcgtgctggg nttcccatca 540
 actcctcacg ctctagttca ccgtaaanna naaaaaactg cgcaaccct caactaaatc 600
 ctgctcaatt gggcgtgggt gaac 624

<210> 7
 <211> 630
 <212> DNA
 <213> Artificial Sequence

<220>
 <223> Mouse rhodopsin hybrid cDNA with a T-->C change at nucleotide 190

<220>
 <221> misc_feature
 <222> (1)..(630)
 <223> n is any nucleotide.

<400> 7
 nnnntcttcc nctttcgttt gttgnanant cannaaan an aggcgncccg gaaggtgtca 60
 gtgcctggag ttgcgctgtg ggacccgtca ntggctgagc tcgccaagca gccttggctc 120
 ctgtctacga agagcccggtg gggcagcctc gagagccgca gccatgaacg gcacagaggg 180
 ccccaatttc tatgtgccct tctccaacgt cacaggcgtg gtgcggagcc ccttcgancn 240

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tccgcagtag tacctggcgg aaccatggca gttctccatg ctggcagcgt acatgttcct 300
gctcatcgtg ctgggcttcc ccatcaactt cctcacgctc tacgtcaccg tacagcacia 360
gaagctgcgc acacccccctc aactacatcc tggctcaact tgggccgntg ggnttggaac 420
ctccttccca ttgggtontt cccggaangg antncaccaa ccacccctct aacacatcaa 480
ctcccatggg ctacttcgtt cttttggggc ccncaggctg ttaatctcga agggcttctt 540
tgccacacct tggaagtga atcncctgt ggttccttg tggctntggc cattaacgct 600
acttgtggtc ctgcaacca ataacaattc 630

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<210> 8
<211> 649
<212> DNA
<213> Artificial Sequence

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<220>
<223> A hammerhead ribozyme (termed Rz33) cloned in pCDNA3

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<220>
<221> misc_feature
<222> (1)..(649)
<223> n is any nucleotide.

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<400> 8
tcccctnntt tttgtagcnc tgccaanaaa aaaggccagc tcacaggana antananaac 60
ccactgctta ctggcttanc naaattaata cgactcacta tagggagacc caagcttggc 120
acatctgatg agtccgtgag gacgaaaaaa ttggtctaca gggccctatt ctataatgtc 180
acctaaatgc tanagctcgc tgatcatcct onactgtgcc ttctacttgc cagcctctn 240
ttgtttgcc cccccctg ccttccttga ccctggaagg tgccactccc actgtccttt 300
cctaataaaa tgaggaaatt gcatcgatt gtctgagtaa gtgtcattct attctggggg 360
gtgggggtgg gcaggacnnc aaaggggaag attgggaaat acaatancca aggancnctc 420
ccccnggta attgcggatt nggtctntc gcttccttaa ggcnгааana aacaactngg 480
gcgctncggg gtttccccn ccncctnt tagcngcga ttantcgccg cgggtgttgt 540
tgttactccc cactnaacg ctacanttgc cagcgcttaa cccccccct tncntttctt 600
ccctccttcc tncacttcc ccggtttcc cnccaancc naaatcngg 649

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<210> 9
<211> 681
<212> DNA
<213> Artificial Sequence

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<220>
<223> Human peripherin cDNA cloned in pCDNA3

<220>
<221> misc_feature
<222> (1)..(681)
<223> n is any nucleotide.

<400> 9
nnttggtggt ncagtnnggat gtctatataa gcagagnctc tggctaacta gnagaaccca 60
ctgcttactg gcttatcgaa attaatacga ctactatag ggagacccaa gcttggtacc 120
gagctcngat ccactagtaa cggccgccag tgtgctggaa ttcttcagcg cccacgacca 180
gtgactatcc cctgctcaag ctgtgattcc gagaccctg ccaccactac tgcattcacg 240
ggggatccca ngctaattggg actcgacatg ggttgcccc acggcanctc cctacanctt 300
gggccanctn cacttttccc aaagnccaa atctccgcct ctgggtcnt taangttnng 360
ggtgggganc tgtgctgtgg gaaacaaccc agaananact tgggcagcat ggngctactg 420
aaagtncatt ttgaacagaa naaacggtcc antttggccc aaggnncnng ntcctaaant 480
ggttctcct ntttggtngn ntcncnctt tccnctngg aatgttcctg aaaaattnaa 540
cnccaaaaaa gaacaaattg aaaaatantt ctnaaaaccc ttttgtncc cccccccna 600
aaagggaagg ggnnggnncc ttttnttec cccccgggg ggggaaaatt tnnnnaanc 660
ccccccccc ccttttttn a 681

<210> 10
<211> 612
<212> DNA
<213> Artificial Sequence

<220>
<223> Human peripherin hybrid DNA with a A-->G change at nucleotide 332

<220>
<221> misc_feature
<222> (1)..(612)
<223> n is any nucleotide.

<400> 10
ttatacnaca cactatangg agaccaagct tggtagcgag ctggatcca ctagtaacgg 60
ccgccagtgt gctggaattc ttcancgcc aggaccagga ctatcccctg ctcaagctgt 120
gattccgaga ccctgccac cactactgca ttcacggggg atcccaggct agtgggacnc 180

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gacatgggta tccccaggg cagctcccta cagcttgggc catctgcact tttcccaagg 240
ccctaagtct ccgcctctgg gctcgtaan gtntgggggtg ggagctgtgc tgtgggaaac 300
aaccgggact acacttggca agcatggcgc tgctgaaagt caagtttgaa cagaaaaaan 360
gggtcaagtt ggccaaggg ctctgggtca gggaaactgg gttccccncc nngttttngg 420
tttgngtgca tcanctncca aaaanannnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn 480
nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn 540
nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn nnnnnnnnnn 600
nnnnnnnnnn nn 612

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<210> 11
<211> 20
<212> DNA
<213> Artificial Sequence

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<220>
<223> Forward 257 mutation primer

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<400> 11
catggcgctg ctgaaagtca 20

```

```

<210> 12
<211> 20
<212> DNA
<213> Artificial Sequence

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<220>
<223> Forward 359 mutation primer

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```

<400> 12
catcttcagc ctgggactgt 20

```

```

<210> 13
<211> 610
<212> DNA
<213> Artificial Sequence

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<220>
<223> A second human peripherin hybrid DNA with a A-->G change at nucleotide 468

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<220>
<221> misc_feature
<222> (1)..(610)
<223> n is any nucleotide.

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<400> 13
ttttnttggg tntcnaatta atacgactca ctatagggag acccaagctt ggtaccgagc 60
tcggatccac tagtaacggc cgccagtgtg ctggaattct tcancgcca ggaccaggac 120
tattccctgc tcaagctgtg attccgagac cctgccacc actactgcat tcacggggat 180
cccaggctag tgggactcga catgggtagc cccaggga gtcacctaca gcttgggcca 240
tctgcacttt tcccaaggcc ctaagtctcc gcctctgggc tcgttaaggt ttggggtggg 300
agctgtgctg tgggaagcaa cccggactac acttggaag catggcgcta ctgaaagtca 360
agtttgacca gaaaaancgg gtcaagttgg gcccaagggc tctgggctcn atgnaaacct 420
nggtttcccc cccctnttt gggctgggca tcactatctt tcagcctggg antgttctg 480
aanattgaac tcccaaagag ancgatgtga tgaataattc tgaaanccat tttgtgcccc 540
actcattgan aaggangggg tgnatcctgt ttcttcactc cctgntggaa aatgctacaa 600
nccctgaacc 610

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<210> 14
<211> 679
<212> DNA
<213> Artificial Sequence

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<220>
<223> Hammerhead ribozyme (termed Rz30) cloned in pCDNA3

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<220>
<221> misc_feature
<222> (1)..(679)
<223> n is any nucleotide.

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<400> 14
cnttgggtgt nctgtcggnt gtctatataa gcagagctct ctggctaact agaagaaccc 60
actgcttact ggcttatcga aattaatacg actcactata gggagaccca agcttacttt 120
cagctgatga gtccgtgagg acgaaagcgc catctagagg gccctattct atagtgtcac 180
ctaaatgcta gagctcgtg atcagcctcg actgtgcctt ctagtgtcca gccatctgtt 240
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taataaaatg atgaaattgc atcgattgt ctgagtaggt gtcattctat tctggggggg 360
gggtggggca ngacancaag ggggaagatt gggaaaacaa tccccgctg ctgggggatgc 420
gggtgggctct atggcttctg aggcgaaana acnctgggg tctngggggg tccncccc 480
ctgtnnccgc ctnnannccg ggggtttgtg ntccccccnc ttancnntnn ttnnnnnncc 540

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nnccccccnnc nntncnnttn ntccnnnnnn tnncnnntt nnnnngntc cnnnnnnnt 600
 nnnnnngggc ncnnnngntc cntnnnncc ncnnnnnn ncnnnnnnn nntntgnngg 660
 cccnnnnenn nnnnnncn. 679

<210> 15
 <211> 691
 <212> DNA
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<220>
 <223> Hammerhead ribozyme (termed Rz31) cloned in pCDNA3

<220>
 <221> misc_feature
 <222> (1)..(691)
 <223> n is any nucleotide.

<400> 15
 nntttntcct acgnccgttt taaananaac cagaccctct gganaattan atnnccactg 60
 ctactggct tatcgaaatc aatacgactc actatangga gaccaagct tacagtccct 120
 gatgagtccg tgaggacgaa aggctgaatc tanagggccc tattctatag tgtcacctaa 180
 atgctagagc tcgctgatca gcctcgactg tgccttctaa ttgccagcca tctgttgttt 240
 gcccctcccc cgtgccttcc ttgaccctgg aaggtgccac tccactgtc ctntcctaata 300
 aaaatgatga nnttgcatcg cattgtctga gtaagtgtca ntctattctg gggggtggg 360
 tggggcanga cancaagggg gaagattggg aaaaacattn cacgcatgcc ggggatgcg 420
 gtgggctctn ttngcntcng aaggcngaaa aaaacnactg ggccctang ggtnncccn 480
 tccccntgt aacngnctt naacnccggg gttgtggtt nccnancct ancctnaac 540
 ttccnncccc nnnccccnc tcttcccttt tctccatc tccnntttt cccgntctcc 600
 cttncactna aatgggggcc cctacnggn ctntntntct cttnnnnccn cccccnana 660
 natatnctng ntnnttcncc tctcgcccc t 691

<210> 16
 <211> 805
 <212> DNA
 <213> mammalian

<220>
 <221> misc_feature
 <222> (1)..(805)
 <223> n is any nucleotide

<220>
 <221> misc_feature
 <223> Human collagen 1A2 (B)

<400> 16
 ntcncgncat ttaancaggc caggncctacc gcnnnggtcca ngtaggccgg gagccccagc 60
 aacgccggga aggccagcag cacccttggc accagtaagg ccgtttgctc caggattacc 120
 angaggtcca acggggccgg agaggcctgg aanaccactt caccacgggg aaccggcggg 180
 tccagtagga ccagcggtac caacagctcc aatttcaccc ttggggccag gggcacctgg 240
 gaagcctgga nggccagcag accaatggga ccagcaggac cacggaccac acttccatca 300
 ctgctttngc ncagctgggc aagggcacia cacttctctc tcacangaac ccacggctcc 360
 tgtttnactg aattccattt cacagggcac agttcacctt cacacaagaa cacgngtgct 420
 cttcatcatc agacatgttt ccctaagtct tgagcagant cagattcagg aaacacacac 480
 ctttgtccac atctctncac agtctcggtt tcaggtacac tccacctgc agaggcactg 540
 accaacctga gacattgaca ttncagncca cagtctgaac tgagcgggca cgccatggcn 600
 agtcatacct gtcagnatca tcttctctta ncattcccaa ngggcagaat gaaagctgac 660
 tccccaatgt cttattttta annanggttt naaanaannn nnnnnnnnnn nnnnnnnnnc 720
 cccccccctt tngggtttat tatctatncc ncccntngga tatctttnc cctttncccc 780
 ctnaaanttt tntttttttt tnnnn 805

<210> 17
 <211> 797
 <212> DNA
 <213> mammalian

<220>
 <221> misc_feature
 <222> (1)..(797)
 <223> n is any nucleotide

<220>
 <221> misc_feature
 <223> Human collagen 1A2 (A)

<400> 17
 ccctttaaaa canggccagg aataccgagg ggtccaggga ggccgggacc ccancaacgc 60
 cgggaangcc cagcagcacc cttggcacca gtaangccgt ttgctccagg attaccagga 120

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ggccaacgg ggccggagan gcctggaaga ccacttcacc acggggaacg gcgggaccag 180
cangaccage gttaccaaca gctccaattt cacccttggg gccaggggca cctgggaagc 240
ctgganggcc agcagaccaa tgggancagc aggaccacgg gaccacactt ccactnctgc 300
cncctggcacc agctgggcaa gggcacaaca cttctctctc acnaagaacc cacggnctct 360
gtttaactga attccatttc acagggcaca gttcaccttc anacagaaca cgggtgtctt 420
tcacatcaa acatntttcc tatnccttga gcagaatcag attcaggaac acacactttg 480
tcacatctcc tcacagtctc ggtttcaggt aacactcnca cctgcagagg cactgacnaa 540
nctcaganat ttanattccn ctcncagtt tgaacttagg cgggccctnn catttggnnt 600
gtcctaacct ntnggggggtt ttncctnnnn nnnnnntttt nacnantccc aanggggana 660
ananagtga ctcctatgtc ttntntnaa aaggtttttt aaaaattaac cccccctn 720
ttgggttatt ttttttttt nccccctt ttngaanen tnnccccntt ttccccnna 780
aanttttttn ttttttt 797

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<210> 18
<211> 697
<212> DNA
<213> Artificial Sequence

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<220>
<223> A hammerhead ribozyme (termed Rz907) cloned in pCDNA3

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<220>
<221> misc_feature
<222> (1)..(697)
<223> n is any nucleotide

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<400> 18
nctttcnntc tnatncatan aagcaggccc tctnnaaaaa ctanantttc cactgcttac 60
tggcttatcg aaancaatac gactcactat agggagaccc aagcttcggc ggctgatgag 120
tccgtgagga cgaaaccagc atctagaggg ccctattcta tagtgtcacc taaatgctag 180
agctcgctga tcagcctcga ctgtgccttc tagttgccag ccactctgtt tttgccctc 240
ccccgtgect tccttgaccc tggaagggtc cactccact gtcctttcct aataaaatga 300
ngaaattgca tcgcattgtc tgagtangtg tcattctatt ctgggggggtg ggggtgggca 360
ngacancaag ggggaagatt gggaanacaa taacaggcat gctggggatg cgggtgggctc 420
tatggcttct gaggcggaaa gaaccaactg gggctctang gggatatccc acnccccgtt 480
taccggcgca ttaancgagg ggggtgtgtg gttaccnca acttaacgct acacttgcca 540

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cgcctaacgc ccttcctttc gcttcttcttct tcttctctcc acttccccgn tttcccttca 600
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